Development of a SYF2 antisense oligonucleotide (ASO) treatment for ALS

Grant Award Details

Development of a SYF2 antisense oligonucleotide (ASO) treatment for ALS

Grant Type: Quest - Discovery Stage Research Projects

Grant Number: DISC2-12158

Project Objective: To complete studies supporting the declaration of a lead a lead SYF2 ASO candidate for the treatment of ALS that is ready for translational stage activities.

Investigator:

Name:	Justin Ichida
Institution:	University of Southern California
Type:	PI

Disease Focus: Amyotrophic Lateral Sclerosis, Neurological Disorders

Human Stem Cell Use: iPS Cell

Award Value: $222,300

Status: Active

Grant Application Details

Application Title: Development of a SYF2 antisense oligonucleotide (ASO) treatment for ALS

Public Abstract:

Research Objective

We will develop an antisense oligonucleotide, or DNA therapy for diverse forms of amyotrophic lateral sclerosis (ALS).

Impact

ALS is fatal and incurable, and if successful, we will develop a treatment that slows or stops ALS progression across a broad range of patients.

Major Proposed Activities

- Selection of the lead drug by testing several candidates for efficacy and safety on ALS patient-derived nerve cells.
- Confirmation that the lead drug is effective and stable in mice.
- Confirmation that the lead drug is safe in mice.

Statement of Benefit to California:	ALS is a fatal, incurable disease and California has one of the highest number of ALS patients of any state. By testing our drug on stem cell-derived nerve cells from Californian ALS patients, we will increase the chances that it will be effective on the types of ALS patients found in California. If successful, our drug will substantially slow or stop ALS disease progression.

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